



INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(51) International Patent Classification 7 :

C12N 15/12, 15/57, 15/67, 15/85, 9/64,
C07K 14/72, C12Q 1/68, A61K 48/00

A1

(11) International Publication Number:

WO 00/49147

(43) International Publication Date:

24 August 2000 (24.08.00)

(21) International Application Number: PCT/EP00/01368

(22) International Filing Date: 18 February 2000 (18.02.00)

(30) Priority Data:

199 07 099.7

19 February 1999 (19.02.99)

DE

60/120,848

19 February 1999 (19.02.99)

US

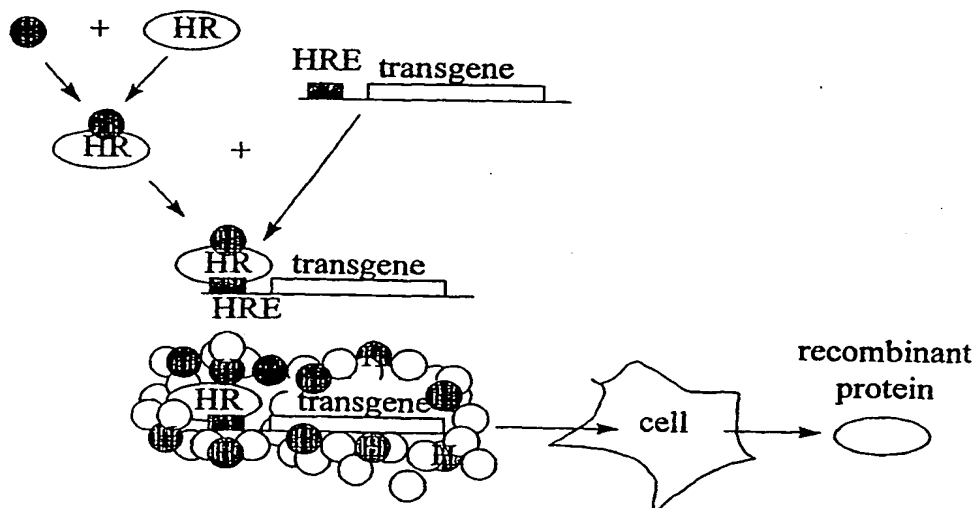
(71) Applicant (for all designated States except US): THERAGÈNE
BIOMEDICAL LABORATORIES GMBH [DE/DE]; Am
Klopferspitz 19, D-82152 Martinsried (DE).

(72) Inventor; and

(75) Inventor/Applicant (for US only): HAUSER-FUNKE, Char-
lotte [DE/DE]; Romanstr. 95, D-80369 München (DE).(74) Agents: HELBING, Jörg et al.; von Kreisler Selting Werner,
Deichmannhaus am Dom, D-50667 Köln (DE).(81) Designated States: AE, AL, AM, AT, AU, AZ, BA, BB, BG,
BR, BY, CA, CH, CN, CR, CU, CZ, DE, DK, DM, EE,
ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP,
KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA,
MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU,
SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, TZ, UA, UG,
US, UZ, VN, YU, ZA, ZW, ARIPO patent (GH, GM, KE,
LS, MW, SD, SL, SZ, TZ, UG, ZW), Eurasian patent (AM,
AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT,
BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU,
MC, NL, PT, SE), OAPI patent (BF, BJ, CF, CG, CI, CM,
GA, GN, GW, ML, MR, NE, SN, TD, TG).

Published

— With international search report.

Before the expiration of the time limit for amending the
claims and to be republished in the event of the receipt of
amendments.(54) Title: HORMONE-HORMONE RECEPTOR COMPLEXES AND NUCLEIC ACID CONSTRUCTS AND THEIR USE IN GENE
THERAPY

(57) Abstract

The invention relates to the use of a nucleic acid construct comprising at least one hormone responsive element and a transgene for preparing an agent for gene transfer. It further relates to particular nucleic acid constructs comprising at least one hormone responsive element and a transgene, wherein one of said at least one hormone responsive elements is not functionally linked to the transgene, vectors comprising such nucleic acid constructs and compositions of matter comprising such nucleic acid constructs wherein the hormone responsive elements of the constructs are coupled to a hormone-hormone receptor complex. The nucleic acid constructs, plasmids, and compositions of matter of the invention have applications in gene therapy, particularly in the treatment of human blood clotting disorders, such as hemophilia. They may also be used to up- or down-regulate target genes and for the delivery of vaccines.